Patient-reported Outcomes Measures

CRT Plenary Meeting

“Developing partnerships between patients, health professionals and the European Society of Cardiology”

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Agenda

• Why patient-reported outcomes measures?
• Choice of tools and methods
• PROMS in:
  – Clinical trials
  – Registers
  – Clinical practice

“PROMs are precisely the missing link in defining a good outcome. They capture quality-of-life issues that are the very reasons that most patients seek care.”
Nothing about me without me.
— Valerie Billingham, Through the Patient's Eyes, Salzburg Seminar Session 356, 1998

«Nothing about me without me»

(Barry MJ & Edgman-Levitan S, NEJM 2012: 366; 9.)
Translational medicine

The importance of patient-reported outcomes: a call for their comprehensive integration in cardiovascular clinical trials

Stefan D. Anker¹,²*, Stefan Agewall³, Martin Borggreve⁴,⁵, Melanie Calvert⁶, J. Jaime Caro⁷, Martin R. Cowie⁸, Ian Ford⁹, Jean A. Paty¹⁰, Jillian P. Riley¹¹, Karl Swedberg¹²,¹³, Luigi Tavazzi¹⁴, Ingela Wiklund¹⁵, and Paulus Kirchhof¹⁶
Box 1 Summary of group recommendations to advance patient-reported outcomes in cardiovascular medicine

- Patient-reported outcomes reflect a key dimension of overall disease burden, and they should be a primary aim of disease management to improve patient well-being.
- Publication of high-quality research papers that describe the development and validation of PRO instruments or that report results of studies where PRO measures were primary or secondary endpoints should be encouraged.
- Patient-reported outcomes measures should be reported in all trials alongside mortality/morbidity outcomes (i.e. as major secondary endpoints) in accordance with the CONSORT PRO Extension.
- Patient-reported outcomes should be available and considered for future practice guidelines.
- Train physicians in the application and interpretation of PROs. Patient-reported outcomes should inform clinical decisions and evidence-based guidelines.
The 6Ds model of health outcomes

(Radosevich DM and Werni TLK. A practical guidebook for implementing, analyzing, and reporting outcomes measurement. Health Outcomes Institute, 1996, p. 1–10.)
The 6Ds model of health outcomes

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QALY

The Quality-Adjusted Life-Year (QALY)

QALY: attempts to represent the impact a therapy has on the length of life while also taking into account any changes in the health-related quality of life (HRQoL). HRQoL is calculated on a scale where 0 = ‘death’ and 1 = ‘perfect’ health (the scale also allows for negative scores).

EUPATI
SF-6D
EQ-5D
Figure 3  Global use of quality-adjusted life years to inform reimbursement decisions. Courtesy: Jaime Caro.
Levels of PROMS

Overall assessment of well-being

Broad domains

Components of each domain

Global items

Generic instruments

Disease specific

(Adapted from Spilker B 1996)
Levels of PROMS

Global items

Generic instruments

Disease specific

(Adapted from Spilker B 1996)
Electronic health (eHealth) literacy

• How to describe and evaluate users’ digital capabilities and experiences?

• PROMS for eHealth literacy:
  – eHEALS (Normann and Skinner 2006)
    • 8 items; combined knowledge, comfort, and perceived skills at finding, evaluating, and applying electronic health information to health problems
  – eHealth Literacy Questionnaire (Kayser et al 2018)
    • 35 items; attributes of users, users ad technologies, and users experience of systems
Measurement Properties of Outcome Measurement Instruments

**RELIABILITY**
- Reliability (test-retest, inter-rater, intra-rater)
- Measurement error (test-retest, inter-rater, intra-rater)
- Internal consistency

**VALIDITY**
- Content validity
  - Face validity
- Criterion validity
  - Concurrent validity, predictive validity
- Construct validity
  - Structural validity
  - Hypotheses-testing
  - Cross-cultural validity

**RESPONSIVENESS**
- Interpretability
- Responsiveness

COSMIN Taxonomy
Systematic reviews of PROMs

• The number of systematic reviews of PROMs is increasing

• COSMIN guidelines for systematic reviews of PROMS (Prinsen CAC et al. Qual Life Res 2018;27:11447-1157)
Clinical outcome endpoints in heart failure trials: a European Society of Cardiology Heart Failure Association consensus document


Chronic heart failure:

“...QoL & PROs... provide insight into treatment effects from the patient’s perspective. The therapeutic goal in HF patients is not limited to prolonging survival; improving the QoL is equally important.” (p. 1089)

Acute heart failure:

Consensus among HF experts

- More research is needed to develop robust methods for capturing HF events other than hospitalization or death
- Patient-reported outcomes are independent endpoints, not surrogates for mortality
- Instruments should be self-administered when possible

Data collection of PROMS

- Apps may be equivalent to other delivery modes such as paper, laptops and SMS
- May result in more complete datasets compared to paper
- Faster completion times?
- Response rates?
- Data accuracy?
- Not enough evidence to make clear recommendations about the impact of apps

(Marcano Belisario et al, Cochrane Library, 2015, Issue 7.)
Computer Adaptive Testing

• The next question is generated from the response of the previous question
• A new final score is estimated at every step (new response)

http://www.healthmeasures.net/explore-measurement-systems/promis
Individual CAT report

Computerized Adaptive Test (CAT) Report

Date: 07-Aug-17
Your age: 44
Your gender: Female
Computerized Adaptive Tests: Fatigue

Your scores for the CATs you completed are shown below.

The diamond is placed where we think your score lies. This diamond is placed on your T-Score, which is a standardized score that is based on an average score of 50, based on responses to the same questions in the United States general population. The T-score also has a standard deviation of 10 points, so a score of 40 or 60 represents a score that is one standard deviation away from the average score of the general US population.

The Standard Error (SE) is a statistical measure of variance and represents the possible range of your score. The lines on either side of the diamond in your profile report show the possible range of your actual score around this estimated score. It is very likely that your score is in the range of these lines.

<table>
<thead>
<tr>
<th>Your Score</th>
<th>SE</th>
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<tbody>
<tr>
<td>Fatigue</td>
<td>44</td>
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Your score on the Fatigue CAT is 44. The average score is 50. Your score indicates that your level of Fatigue is higher (worse) than:

- 30 percent of people in the general population
- 26 percent of people age 35-45
- 24 percent of females
SRH predict survival in Norwegian HF clinics

• N=3632 (n=1778 for SRH)
• 24 hospital outpatient clinics
• MLHFQ in tertiles was an independent predictor of mortality

(Hole T. et al, Eur J Heart Failure. 2010, 12; 1247-1252)
Figure 1. Clinical feedback system. Graphic score presentation of the Minnesota Living with Heart Failure Questionnaire (lower scores indicates better health) integrated into the patient medical records in order to be used by the patient and the cardiac team during the clinical encounter in a heart failure clinic.

2015 ESC Guidelines for the management of patients with ventricular arrhythmias and the prevention of sudden cardiac death

The Task Force for the Management of Patients with Ventricular Arrhythmias and the Prevention of Sudden Cardiac Death of the European Society of Cardiology (ESC)

Endorsed by: Association for European Paediatric and Congenital Cardiology (AEPC)

Authors/Task Force Members: Silvia G. Priori* (Chairperson) (Italy), Carina Blomström-Lundqvist* (Co-chairperson) (Sweden), Andrea Mazzanti† (Italy), Nico Blom‡ (The Netherlands), Martin Borggrefe (Germany), John Camm (UK), Perry Mark Elliott (UK), Donna Fitzsimons (UK), Robert Hatala (Slovakia), Gerhard Hindricks (Germany), Paulus Kirchhof (UK/Germany), Keld Kjeldsen (Denmark), Karl-Heinz Kuck (Germany), Antonio Hernandez-Madrid (Spain), Nikolaos Nikolaou (Greece), Tone M. Norekval (Norway), Christian Spaulding (France), and Dirk J. Van Veldhuisen (The Netherlands)
Psychosocial impact of ICD treatment

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<th>Levelb</th>
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- Controlled defibrillator trials demonstrated preserved or improved QoL in recipients of a defibrillator compared with that in controls.
- Anxiety (8–63%) and depression (5–41%) common in defibrillator patients, most pronounced in patients experiencing inappropriate and/or frequent shocks (e.g. >5 shocks).
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Summary

• PROs are important and should be used in research, guidelines and clinical practice
• Choose valid and reliable measurement tools
• Choose relevant measurement tools; patient involvement
• Combination of generic and disease specific questions
• Take health literacy and ehealth literacy into account